PRPE-SF, polarized hESC-derived RPE Soluble Factors, as a Therapy for Early Stage Dry Age-related Macular Degeneration

Grant Award Details

PRPE-SF, polarized hESC-derived RPE Soluble Factors, as a Therapy for Early Stage Dry Age-related Macular Degeneration

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-11532

Investigator:

Name:	Mark Humayun
Institution:	University of Southern California
Type:	PI

Disease Focus: Age-related macular degeneration, Vision Loss

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $3,733,556

Status: Pre-Active

Grant Application Details

Application Title: PRPE-SF, polarized hESC-derived RPE Soluble Factors, as a Therapy for Early Stage Dry Age-related Macular Degeneration

Public Abstract:

Translational Candidate

PRPE-SF is a preparation of soluble factors from polarized retinal pigment epithelial cells, to support survival of photoreceptors in dry AMD (dAMD).

Area of Impact

dAMD with early geographic atrophy (RPE dysfunction/photoreceptor degeneration) that does not involve the fovea, with visual acuity better than 20/80.

Mechanism of Action

PRPE-SF is composed of multiple neuroprotective and anti-inflammatory factors. The cause of AMD is multifactorial, with both genetic & environmental components. However, not all factors have been defined & targeting the known factors (ex. complement pathway) has not been successful. PRPE-SF does not target one specific mechanism as its multitude of factors may work synergistically (paracrine effect) to provide an optimal microenvironment for photoreceptor survival & function.

Unmet Medical Need

AMD affects over 2 million people nationwide (90% dAMD). The target population for PRPE-SF is patients with dAMD with early geographic atrophy, intended to slow progression of disease. There are no products approved for this target & successful development of PRPE-SF would be a major breakthrough.

Project Objective

To enable an FDA pre-IND meeting for PRPE-SF.

Major Proposed Activities

- Manufacturing Process Dev.
  •Finalize manufacturing for transfer to cGMP
  •Develop release testing analytics
  •Scale PRPE-SF for Phase 1 clinical trial
- Preclinical Dev.
  •Assess activity, dose & dose regimen for Phase 1 clinical trial
  •Examine pilot distribution & safety of final PRPE-SF drug product.
- Clinical Trial Planning.
  • Develop clinical plan & protocol synopsis for clinical trial
  • Hold interact meeting with FDA
  • Hold pre-IND meeting with FDA

Statement of Benefit to California:

AMD is one of the most common causes of blindness in those 50 or older with an estimated 400,000 Californians projected to suffer from AMD by 2020. AMD is a debilitating disease, which results in loss of independence and productivity, increased injury and dramatic decline in quality of life. With a $3 billion economic burden annually in California, PRPE-SF will be developed by California based companies, creating additional jobs for Californians and a treatment for this devastating disease.
